CIRM Funded Clinical Trials

A monoclonal antibody that depletes blood stem cells and enables chemotherapy free transplants

Disease Area:	X-linked Severe Combined Immunodeficiency (X-linked SCID)
Investigator:	Judith Shizuru
Institution:	Stanford University
CIRM Grant:	DR2A-05365
Award Value:	$19,068,382
Trial Sponsor:	Stanford University
Trial Stage:	Phase 1/2
Trial Status:	Recruiting
Targeted Enrollment:	90
ClinicalTrials.gov ID:	NCT02963064

Judith Shizuru

Details:
This trial proposes to replace SCID patients' dysfunctional immune cells with healthy ones using a safer form of bone marrow transplant (BMT). Current BMT procedures must use toxic chemotherapy to make space in the bone marrow for the healthy transplanted stem cells to engraft. The Stanford team will instead test a safe, non-toxic protein called a monoclonal antibody that targets and removes the defective blood forming stem cells. The hope is that this treatment will promote the engraftment of the transplanted stem cells in the patient. If successful, the procedure could open up similar BMT therapies to patients with other auto-immune diseases such as multiple sclerosis, lupus or diabetes that are generally not candidates for BMT currently.

Design:
Open label, dose escalation trial.

Goal:
Safety. Dosing. Efficacy - HSC engraftment, immune reconstitution.

Updates:
Enrolling.

Contact Trial Sponsor

Source URL: http://www.cirm.ca.gov/clinical-trial/monoclonal-antibody-depletes-blood-stem-cells-and-enables-chemotherapy-free